Asterias Biotherapeutics announces positive 12-month results for CIRM-funded spinal cord injury trial

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October 2nd, 2017, Oakland, CA – Asterias Biotherapeutics today announced that four of six patients with spinal cord injuries, who each had 10 million stem cells transplanted at the injury site, have shown encouraging improvement in their ability to move their hands and arms 12 months after their treatment.

The results are part of the second group of patients treated in the SCiStar study, which is being funded by the California Institute for Regenerative Medicine (CIRM). The trial is testing the safety and effectiveness of three separate doses of a stem cell-based therapy called AST-OPC1. This is for people who have sustained serious spinal cord injuries high up in the neck, at the C-4 to C-7 level.

In a news release, Edward Wirth, M.D. Ph.D., Chief Medical Officer of Asterias, said: "The 12-month results suggest that many of the Cohort 2 subjects are seeing a durable clinical benefit above and beyond what you would expect to see from spontaneous recovery following a severe spinal cord injury. Our target for the entire SCiStar study since its commencement was that at 12 months 45-50% of study subjects would recover at least 2 motor levels on at least one side. We are very encouraged that Cohort 2 has exceeded this target recovery rate."

Patients in the study are treated with a type of cell called an oligodendrocyte progenitor. These cells – which are derived from embryonic stem cells - are important support cells that protect the damaged nerve cells in the spinal cord.

Asterias is now working with other groups of patients, testing a dose of 20 million stem cell and also testing this approach on patients with less severe injuries.

"At CIRM, our mission is to support promising stem cell therapies that could help treat challenging conditions like spinal cord injury, so we are encouraged by this preliminary clinical data from Asterias," said Maria Millan, M.D., President and CEO of CIRM.

Asterias also announced that AST-OPC1 has been granted Regenerative Medicine Advanced Therapy (RMAT) designation by the US Food and Drug Administration (FDA). RMAT, a provision under the 21st Century Cures Act, means that AST-OPC1 will be eligible for priority review and accelerated regulatory approval if the treatment continues to show that it is both safe and effective for treating spinal cord injuries.

"We are very pleased that Asterias has received RMAT designation," said Dr. Millan. "Because this trial tests a stem cell approach for cell replacement and repair it is particularly well suited for the RMAT designation. This is the third of CIRM's clinical programs to obtain RMAT designation. Our Agency is in the business of acceleration, and we view this as an important way to collaboratively work with the FDA to advance the stem cell regenerative medicine field to address a serious unmet medical need."

CIRM has funded a total of 40 clinical trials since its inception, seven of which target challenging indications in the central nervous system including Amyotrophic Lateral Sclerosis (ALS), spinal cord injury, and degenerative eye diseases.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to www.cirm.ca.gov.

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